



NEWS DIGEST

- Landmark legislation in Italy: Provisions for the treatment of rare diseases and for the support of research and production of orphan drugs
- FDA approves first drug to improve growth in children with most common form of Dwarfism
- NICE creates new menu of treatment options for those suffering from depression

LANDMARK LEGISLATION IN ITALY: PROVISIONS FOR THE TREATMENT OF RARE DISEASES & FOR THE SUPPORT OF RESEARCH & PRODUCTION OF ORPHAN DRUGS

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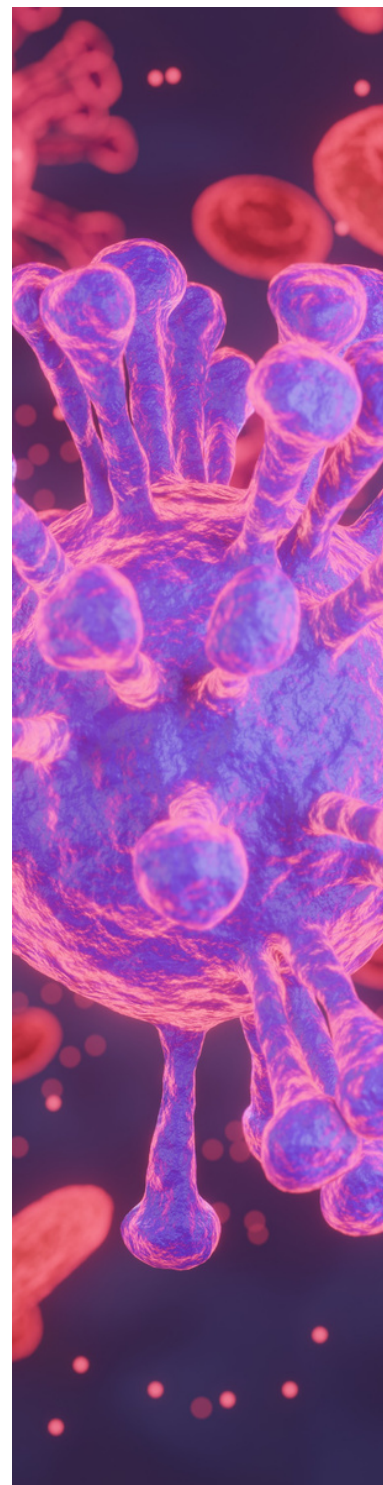
The Testo Unico sulle Malattie Rare (Consolidated Law on Rare Diseases), approved on 3rd November 2021, will provide a comprehensive framework for the management of rare diseases.

First of all, this is the first law dedicated solely to rare diseases, aimed at organizing this area in an orderly and (as much as possible) way, which affects about 2 million people in Italy.

In general, the aims of the provision are: to guarantee the uniformity of diagnostic, therapeutic and assistance care for rare patients throughout the national territory ; systematically and organically regulate the interventions dedicated to supporting research, both on rare diseases and on orphan drugs.

The law also provides for a solidarity fund dedicated to the financing of measures to support the work of care and assistance of people with rare disease who are 100% civil disabled or disabled with a connotation of gravity.

source: <https://bit.ly/3ozlwhz>



FDA APPROVES FIRST DRUG TO IMPROVE GROWTH IN CHILDREN WITH MOST COMMON FORM OF DWARFISM

THE NEWS

The U.S. Food and Drug Administration (FDA) approved Voxzogo (vosoritide) injection to improve growth in children five years of age and older with achondroplasia and open epiphyses (growth plates), meaning these children still have the potential to grow.

Achondroplasia (most common form of dwarfism) is a genetic condition that causes severely short stature and disproportionate growth. The average height of an adult with achondroplasia is approximately four feet. People with achondroplasia have a genetic mutation that causes a certain growth regulation gene called fibroblast growth factor receptor 3 to be overly active, which prevents normal bone growth.

Voxzogo works by binding to a specific receptor called natriuretic peptide receptor-B that reduces the growth regulation gene's activity and stimulates bone growth.

"Today's approval fulfils an unmet medical need for more than 10,000 children in the United States and underscores the FDA's commitment to help make new therapies available for rare diseases," said Theresa Kehoe, M.D., director of the Division of General Endocrinology in the FDA's Center for Drug Evaluation and Research.

source: <https://bit.ly/3lOxQZC>



NICE CREATES NEW MENU OF TREATMENT OPTIONS FOR THOSE SUFFERING FROM DEPRESSION

THE NEWS

People with depression should make the choice on what treatment option is right for them, in discussion with their healthcare professional, NICE says in new draft guidance.

An independent NICE guideline committee has developed the first guideline for 12 years to identify, treat and manage depression in adults.

It has looked at the evidence on the treatment of new depressive episodes, chronic depression, preventing relapse, patient choice, and the organisation of, and access to, mental health services.

The committee has created a menu of treatment options to allow patients to pick the one which is right for them, in a shared decision-making discussion between them and their healthcare practitioner.

Patients with less severe depression could choose from the menu as a first-line treatment option from, for example, cognitive behavioural therapy (CBT), exercise, counselling or psychotherapy.

source: <https://bit.ly/3IPK7wS>



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